



Neurocrine Biosciences Presents Real-World Evidence Mapping the Lifelong, Evolving Impact of Classic Congenital Adrenal Hyperplasia

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- Decades of real-world medical records highlight the diverse challenges of living with classic congenital adrenal hyperplasia and how needs evolve from childhood through adulthood
- Patient records showed growth and bone-age monitoring in childhood; puberty and reproductive health concerns through adolescence and young adulthood; and increasingly diverse clinical needs in adulthood, while treatment and self-management demands persisted across life stages
- The analysis captured impacts beyond traditional medical complications, including daily activities, school, work, caregiving, self-management, and access to care
- Mapping patient experiences over time may help healthcare professionals anticipate evolving needs and inform education, support strategies, and coordination across care teams

SAN DIEGO, Oct. 16, 2026 /PRNewswire/ -- [Neurocrine Biosciences, Inc.](#) (Nasdaq: NBIX) today announced new real-world data demonstrating how the clinical and day-to-day impact of classic congenital adrenal hyperplasia can vary among individuals and evolve throughout a patient's life. The findings will be presented at the Endocrine Nurses Society Annual Symposium 2026, taking place Oct. 16-17 in Phoenix.



The analysis used a novel patient journey mapping approach to combine de-identified electronic medical records from [CAHtalog®](#), a registry of patients with classic congenital adrenal hyperplasia (CAH) in the United States developed by Neurocrine in partnership with the [CARES Foundation](#). CAHtalog was designed to characterize treatment patterns over time, natural history, and impact of CAH. The records used for this analysis spanned decades of care across providers, specialties, and health systems, allowing clinical, treatment, monitoring, and humanistic experiences to be examined within individual patients with CAH.

"Classic congenital adrenal hyperplasia is a complex, lifelong disease, and every person living with CAH experiences the condition differently, with needs that change over time," said Eiry W. Roberts, M.D., Chief Medical Officer, Neurocrine Biosciences. "By connecting decades of real-world medical records into longitudinal patient journeys, this analysis reveals how the impact of the condition accumulates across the lifespan and highlights challenges that might otherwise be missed in traditional clinical assessments. These insights may help healthcare professionals anticipate periods when patients could benefit from additional education, support, and coordination from their care team."

Electronic medical records from 10 adults (four males, six females) with classic CAH in the CAHtalog registry were used to construct aggregated and sex-stratified patient journeys.* The data comprised more than 2,800 CAH-relevant events representing more than 300 patient-years of medical history. Patient journeys were developed using large language model-assisted analysis and were reviewed and validated by the study team.

Patient records reflected a range of clinical and day-to-day challenges across life stages. Impacts emerged in infancy and centered on diagnosis, treatment, managing acute illness, and preparing caregivers for emergency steroid use. In childhood, records reflected growth-related concerns with bone-age monitoring becoming increasingly important. During adolescence, puberty brought new androgen-related symptoms and challenges with staying on treatment, while monitoring hormone and steroid levels became a greater focus. In young adulthood, new reproductive, surgical, and transition-of-care needs emerged. In adulthood, patients experienced a wider range of health concerns and greater need for imaging and testing.

Treatment, monitoring, caregiver support, and self-management demands recurred and evolved across life stages. Glucocorticoid dose adjustments and stress dosing, or temporarily increasing steroids during illness, injury, or other periods of physical stress, were documented over time in all 10 patients. Records also reflected ongoing monitoring and care coordination, including specialty referrals, changes in providers, treatment access, and the transition to adult endocrinology. Treatment management evolved from caregiver-led support to increasing self-management. These findings underscore ongoing education and preparedness needs for patients and caregivers.

Routine clinical records captured substantial challenges beyond medical complications, extending to school, work, and daily functioning. These impacts evolved over time from challenges managing treatment and progressing in sports during childhood to disruptions in education, work, and physical activity later in life.

While many treatment and weight-related impacts were shared across sexes, the analysis also identified certain sex-specific aspects of care. Menstrual concerns were documented among females, and testicular imaging and testicular adrenal rest tumor concerns were documented among males.

"Nurses are uniquely positioned to understand the full experience of living with adrenal insufficiency — not only the laboratory values and clinical data, but how the condition affects patients and families as they navigate daily life, transitions in care, and changing needs over time," said Louise Fleming, Ph.D., MSN-Ed, RN, FAAN, presenting author and Professor of Nursing at the University of Virginia School of Nursing. "Recognizing the broader patient journey can help us anticipate when additional education, stress-dosing preparation, self-management support or care coordination may be needed. It also allows us to move beyond responding to individual clinical encounters toward providing more proactive, coordinated and patient-centered care across the lifespan."

*This descriptive analysis was intended to provide qualitative insights into patient experiences rather than quantitative estimates, with some observations based on one or two individuals. The findings are illustrative and should not be interpreted as prevalence estimates or evidence of causality.

Additional Presentations at the Endocrine Nurses Society Annual Symposium 2026 Include:

- Long-term Treatment with Crinecerfont Enables Sustained Decreases in Glucocorticoid Doses and Improves Clinical Outcomes in Children and Adolescents with Classic Congenital Adrenal Hyperplasia: 2-Year Results from CAHtalyst® Pediatric
- Crinecerfont Improves Cardiometabolic Outcomes in the Context of Sustained Glucocorticoid Dose Reduction: 2-Year Results from the CAHtalyst Adult Study

About Congenital Adrenal Hyperplasia

Congenital adrenal hyperplasia (CAH) is a rare genetic condition that results in an enzyme deficiency that alters the production of adrenal steroid hormones, such as cortisol, aldosterone and adrenal androgens. Severe enzyme deficiency leads to an inability of the adrenal glands to produce enough cortisol and, in approximately 75% of cases, aldosterone. Because individuals with CAH are typically still able to produce androgens, the unused precursors that would normally be used to make cortisol instead result in the production of excess amounts of androgens. If left untreated, CAH can result in adrenal crisis and even death.

Exogenous glucocorticoids (GCs) are necessary to correct the endogenous cortisol deficiency, but historically, doses higher than those needed for cortisol replacement (supraphysiologic) have been used to lower the elevated levels of adrenocorticotrophic hormone (ACTH) and adrenal androgens. However, GC treatment at supraphysiologic doses has been associated with serious and significant complications of steroid excess, including metabolic issues such as weight gain and diabetes, cardiovascular disease and osteoporosis. Additionally, long-term treatment with supraphysiologic GCs may have psychological and cognitive impacts, such as changes in mood and memory. Adrenal androgen excess has been associated with abnormal bone growth and development in pediatric patients, female health problems such as excess facial hair growth and menstrual irregularities, in addition to cardiometabolic and fertility issues in both sexes. The symptoms of high ACTH may include testicular adrenal rest tumors (TARTs).

About the CAHtalog® Registry

In 2021, the CARES Foundation, Neurocrine Biosciences and PicnicHealth partnered to establish the CAHtalog (**C**ongenital **A**drenal **H**yperplasia: **P**atient and **C**linical **O**utcomes in **R**eal-**W**orld **P**ractice **S**ettings) Registry. The CAHtalog Registry is a U.S.-based CAH patient registry, or collection of clinical patient data, for patients with CAH due to 21-hydroxylase deficiency. The database was developed to support patient-centered clinical research to enhance the scientific community's foundational knowledge about CAH, and ultimately help patients who live with the condition. Patients consent to the inclusion of their data in the registry with CAH researchers primarily utilizing de-identified data. For more information about the CAHtalog Registry, please visit [CAHtalog.com](https://cahtalog.com).

About the CAHtalyst® Studies

The Phase 3 CAHtalyst global registrational studies were designed to evaluate the safety, efficacy and tolerability of CRENESSITY® (crinecerfont) in children and adults with classic congenital adrenal hyperplasia (CAH) due to 21-hydroxylase deficiency. The CAHtalyst studies were the largest-ever clinical trial program in classic CAH, including 285 pediatric and adult patients.

The [CAHtalyst Pediatric study](#) included 103 pediatric patients four to 17 years of age. The study tested two questions. The first question evaluated whether four weeks of CRENESSITY treatment could improve androgen control. The second question evaluated whether an additional 24 weeks of CRENESSITY treatment enabled customized glucocorticoid (GC) down-titration while androstenedione levels were maintained or improved.

The [CAHtalyst Adult study](#) included 182 adult patients 18 to 58 years of age. Similarly, the first question of the study evaluated

whether four weeks of CRENESSITY treatment could improve androgen control, and the second question evaluated whether an additional 20 weeks of CRENESSITY treatment enabled GC reduction to physiologic range while androstenedione levels were maintained or improved.

Data from the CAHtalyst Phase 3 studies supported approval of CRENESSITY by the U.S. Food and Drug Administration in December 2024. The open-label extension treatment portions of both studies are ongoing.

About CRENESSITY® (crinecerfont)

CRENESSITY is a potent and selective oral corticotropin-releasing factor type 1 receptor (CRF₁) antagonist that reduces and controls excess adrenocorticotrophic hormone (ACTH) and adrenal androgens through a non-glucocorticoid (GC) mechanism for the treatment of classic congenital adrenal hyperplasia (CAH). Antagonism of CRF₁ receptors in the pituitary has been shown to decrease ACTH levels, which in turn decreases the production of adrenal androgens and potentially the symptoms associated with CAH. The robust clinical study data demonstrate that lowering adrenal androgen levels with CRENESSITY enables lower, more physiologic dosing of GCs to replace missing cortisol.

CRENESSITY comes in capsules and an oral solution. For adults 18 years of age and older, the recommended dosage is 100 mg twice daily taken orally with a meal. For pediatric patients 4 to 17 years of age weighing less than 55 kg (121 lbs), the recommended dosage is based on body weight and is administered twice daily, taken orally with a meal. For pediatric patients weighing more than 55 kg (121 lbs), the recommended dosage is 100 mg twice daily taken orally with a meal. Healthcare providers can work with patients to determine the appropriate formulation for use depending on patient needs. Patients receiving CRENESSITY should continue GC therapy for cortisol replacement.

Important Information

Approved Uses

CRENESSITY® (crinecerfont) is a prescription medicine used together with glucocorticoids (steroids) to control androgen (testosterone-like hormone) levels in adults and children 4 years of age and older with classic congenital adrenal hyperplasia (CAH).

IMPORTANT SAFETY INFORMATION

Do not take CRENESSITY if you:

Are allergic to crinecerfont, or any of the ingredients in CRENESSITY.

CRENESSITY may cause serious side effects, including:

Allergic reactions. Symptoms of an allergic reaction include tightness of the throat, trouble breathing or swallowing, swelling of the lips, tongue, or face, and rash. If you have an allergic reaction to CRENESSITY, get emergency medical help right away and stop taking CRENESSITY.

Risk of Sudden Adrenal Insufficiency or Adrenal Crisis with Too Little Glucocorticoid (Steroid) Medicine. Sudden adrenal insufficiency or adrenal crisis can happen in people with congenital adrenal hyperplasia who are not taking enough glucocorticoid (steroid) medicine. You should continue taking your glucocorticoid (steroid) medicine during treatment with CRENESSITY. Certain conditions such as infection, severe injury, or shock may increase your risk for sudden adrenal insufficiency or adrenal crisis. Tell your healthcare provider if you get a severe injury, infection, illness, or have planned surgery during treatment. Your healthcare provider may need to change your dose of glucocorticoid (steroid) medicine.

Before taking CRENESSITY, tell your healthcare provider about all of your medical conditions, including if you: are pregnant or plan to become pregnant, or are breastfeeding or plan to breastfeed.

Tell your healthcare provider about all the medicines you take, including prescription and over-the-counter medicines, vitamins and herbal supplements.

The most common side effects of CRENESSITY in adults include tiredness, headache, dizziness, joint pain, back pain, decreased appetite, and muscle pain.

The most common side effects of CRENESSITY in children include headache, stomach pain, tiredness, nasal congestion, and nosebleeds.

These are not all the possible side effects of CRENESSITY. Call your healthcare provider for medical advice about side effects. You are encouraged to report negative side effects of prescription drugs to the FDA. Visit MedWatch at www.fda.gov/medwatch or call 1-800-FDA-1088.

Dosage Forms and Strengths: CRENESSITY is available in 50 mg and 100 mg capsules, and as an oral solution of 50 mg/mL.

Please see full [Prescribing Information](#).

About Neurocrine Biosciences, Inc.

Neurocrine Biosciences is a leading biopharmaceutical company with a simple purpose: to relieve suffering for people with great needs. We are dedicated to discovering, developing and commercializing life-changing treatments for patients with under-addressed neurological, psychiatric, endocrine and immunological disorders. The company's diverse portfolio includes FDA-approved treatments for tardive dyskinesia, chorea associated with Huntington's disease, classic congenital adrenal hyperplasia, hyperphagia in Prader-Willi syndrome, endometriosis* and uterine fibroids*, as well as a robust pipeline including multiple compounds in mid- to late-phase clinical development across our core therapeutic areas. For more than three decades, we have applied our unique insight into neuroscience and the interconnections between brain and body systems to treat complex conditions. We relentlessly pursue medicines to ease the burden of debilitating diseases and disorders, because you deserve brave science. For more information, visit [neurocrine.com](https://www.neurocrine.com), and follow the company on [LinkedIn](#), [X](#), [Facebook](#) and [YouTube](#). (*in collaboration with AbbVie)

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Forward-Looking Statements

In addition to historical facts, this press release contains forward-looking statements that involve a number of risks and uncertainties. These statements include, but are not limited to, statements regarding the interpretation and potential relevance of the data described in this press release, including the extent to which patient journey mapping may help healthcare professionals anticipate evolving needs among patients with classic congenital adrenal hyperplasia (CAH) and inform education, patient support and coordination across care teams; the potential benefits to be derived from CRENESSITY for the treatment of CAH; and the value and benefits CRENESSITY brings to patients with CAH. Factors that could cause actual results to differ materially from those stated or implied in the forward-looking statements include, but are not limited to, the following: risks and uncertainties as to whether the data described in this press release, which are based on records from a limited number of patients, are representative of the broader CAH patient population and whether patient journey mapping will help healthcare professionals anticipate evolving patient needs and inform education, patient support and coordination across care teams; risks and uncertainties associated with Neurocrine Biosciences' business and finances in general, as well as risks and uncertainties associated with the commercialization of CRENESSITY, including the extent to which patients and physicians accept and adopt CRENESSITY; whether CRENESSITY receives adequate reimbursement from third-party payors; risks and uncertainties relating to competitive products and technological changes that may limit demand for CRENESSITY; risks associated with the Company's dependence on third parties for development and manufacturing activities related to CRENESSITY, and the ability of the Company to manage these third parties; risks that additional regulatory submissions for CRENESSITY may not occur or be submitted in a timely manner; risks that the FDA or other regulatory authorities may make adverse decisions regarding CRENESSITY; risks that post-approval CRENESSITY commitments or requirements may be delayed; risks that CRENESSITY may be precluded from commercialization by the proprietary or regulatory rights of third parties, or have unintended side effects, adverse reactions or incidents of misuse; and other risks described in the Company's periodic reports filed with the Securities and Exchange Commission, including without limitation the Company's quarterly report on Form 10-Q for the quarter ended June 30, 2026. Neurocrine Biosciences disclaims any obligation to update the statements contained in this press release after the date hereof other than required by law.

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